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EPAR summary for the public

Ferriprox

deferiprone

This document is a summary of the European public assessment report (EPAR) for Ferriprox. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Ferriprox.

What is Ferriprox?

Ferriprox is a medicine that contains the active substance deferiprone. It is available as tablets (500 and 1,000 mg) and as an oral solution (100 mg/ml).

What is Ferriprox used for?

Ferriprox is an 'iron chelator' (a substance that attaches to iron) that is used to treat iron overload (an excess of iron in the body) in patients with thalassaemia major. This is an inherited disease in which patients are unable to make enough haemoglobin, the protein found in red blood cells that carries oxygen around the body.

Ferriprox is used:

- on its own, when standard iron chelator treatment cannot be used or is inadequate;
- in combination with another iron chelator, when treatment with one iron chelator on its own is ineffective or when prevention or treatment of life-threatening conditions (mainly affecting the heart) requires rapid or intensive correction of iron levels.

The medicine can only be obtained with a prescription.

How is Ferriprox used?

Ferriprox treatment should be started and maintained by a doctor who has experience in the treatment of patients with thalassaemia.

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The usual dose of Ferriprox is 75 mg per kilogram body weight each day, divided into three separate doses. Doses above 100 mg/kg a day are not recommended because of a potentially increased risk of side effects. The doctor may adjust the dose of Ferriprox depending on the patient's response, which should be measured every two to three months with blood tests. The doctor may interrupt treatment if iron levels in the body get too low.

For more information, see the summary of product characteristics (also part of the EPAR).

How does Ferriprox work?

Patients with thalassaemia major need frequent blood transfusions. When patients receive repeated transfusions, the transfused red cells bring iron into the body. However, the body does not have a natural way of removing excess iron, so it builds up. Over time, the excess iron can damage important organs such as the heart or liver. The active substance in Ferriprox, deferiprone, is an iron chelator. It attaches to iron in the body to form a compound that can be excreted by the body, mainly in the urine, and to a lesser extent in the stools. This helps to correct the iron overload and prevent damage due to excess iron.

How has Ferriprox been studied?

Ferriprox was initially studied in three studies involving 247 patients over six years of age with thalassaemia major, with the main study comparing Ferriprox with deferoxamine in 71 patients over two years. The study was 'open label', meaning that the doctor and patients knew which medicine they were using, because Ferriprox was given by mouth, whereas deferoxamine was given by injection under the skin overnight. A later study compared treatment alternating Ferriprox and deferoxamine (five days' Ferriprox plus two days' deferoxamine each week) with continuous treatment with deferoxamine on its own, in 60 patients over 12 months.

In all of the studies, the main measure of effectiveness was the change in the levels of ferritin in the blood. Ferritin is a protein that stores iron in the body. The blood ferritin level indicates how much iron is being stored in the body.

In addition, studies from the published literature in patients with thalassaemia major were provided to support use of Ferriprox in combination with another iron chelator.

What benefit has Ferriprox shown during the studies?

In the initial study comparing Ferriprox with deferoxamine, the average blood ferritin levels were similar in the two treatment groups. However, the average iron concentration in the liver of Ferriprox-treated patients seemed to increase more than in deferoxamine-treated patients.

In the alternating treatment study, the treatment schedule combining Ferriprox for five days with deferoxamine for two days reduced blood ferritin levels to the same extent as deferoxamine taken on its own. However, there were too few patients in the study to prove whether or not such a schedule is as effective as deferoxamine taken on its own.

Published studies on use of Ferriprox together with deferoxamine reported greater reductions in blood ferritin levels when both medicines were used in combination compared with using either medicine on its own. In a published study, Ferriprox together with deferoxamine also led to greater decreases in iron in the heart compared with patients taking deferoxamine alone.

What is the risk associated with Ferriprox?

The most common side effects with Ferriprox (seen in more than 1 patient in 10) are reddish-brown urine (showing that iron is being eliminated), nausea (feeling sick), abdominal pain (stomach ache) and vomiting. Less common but more serious side effects are agranulocytosis (very low levels of granulocytes, a type of white blood cell) and neutropenia (low levels of neutrophils, a type of white blood cell). For the full list of all side effects reported with Ferriprox, see the package leaflet.

Ferriprox must not be used in people who have previously had neutropenia repeatedly or agranulocytosis. Ferriprox must also not be used with medicines that might cause neutropenia or agranulocytosis. When taking Ferriprox, the patient's neutrophil count should be checked every week. If the patient gets an infection, treatment with Ferriprox should be temporarily stopped and the neutrophil count checked more often. Patients should tell their doctor immediately if they have symptoms that might be due to an infection, such as fever, sore throat and flu-like symptoms.

Ferriprox must not be used in women who are pregnant or breastfeeding. For the full list of restrictions with Ferriprox, see the package leaflet.

Why has Ferriprox been approved?

The CHMP decided that Ferriprox's benefits are greater than its risks and recommended that it be given marketing authorisation.

What measures are being taken to ensure the safe and effective use of Ferriprox?

Patients taking Ferriprox, or their carers, must be given a reminder card with information on how to take the medicine safely.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Ferriprox have also been included in the summary of product characteristics and the package leaflet.

Other information about Ferriprox:

The European Commission granted a marketing authorisation valid throughout the European Union for Ferriprox on 25 August 1999.

The full EPAR for Ferriprox can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Ferriprox, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 05-2016.